

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-155)

BETIBEGLOGENE AUTOTEMCEL

PRONUNCIATION

be" ti beg' loe jeen aw" toe tem' sel

THERAPEUTIC CLAIM

Treatment of betathalassemia

DESCRIPTION

LentiGlobin BB305 Drug Substance consists of an autologous CD34+ enriched cell population that contains hematopoietic stem cells (HSC's) transduced with the LentiGlobin BB305 lentiviral vector (LVV) encoding for the β^{A-T87Q} -globin gene. The CD34+ enriched cell population containing hematopoietic stem and progenitor cells is obtained from autologous peripheral blood mononuclear cells (PBMCs) collected by apheresis from the patient following mobilization of leukocytes.

SCHEMATIC MAP

Figure 1: Schematic Map of the LentiGlobin BB305 Proviral DNA

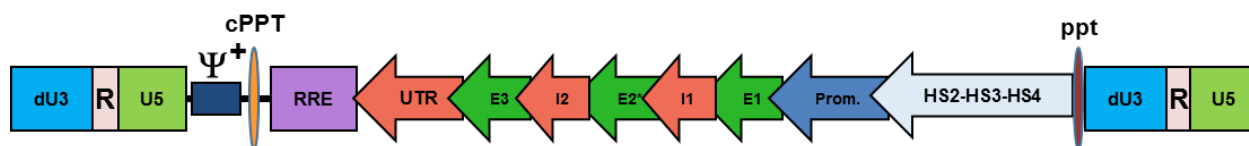


Figure definitions: dU3, promoter deleted unique 3'; R, repeat; U5 unique 5'; Ψ^+ , packaging signal; cPPT, central polypurine tract; RRE, rev responsive element; UTR, untranslated region; E3, Exon3; I2, Intron2; E2*, Exon2 with threonine87glutamine codon; I1, intron1; E1, Exon1; Prom, promoter; HS2-HS3-HS4, hypersensitive site 2,3,4; ppt, polypurine tract.

TRADEMARK

None as yet

SPONSOR

Bluebird Bio

CODE DESIGNATION

LentiGlobin BB305

UNII

MEE8487RTP

WHO NUMBER

10900

SCS